

Memorandum

TO: Martin Erlichman
Senior Health Science Analyst
Agency for Healthcare Research and Quality

FROM: Sean R. Tunis, M.D., M.Sc.
Director
Coverage and Analysis Group
Office of Clinical Standards and Quality

SUBJECT: Request for a Technology Assessment on Liver Transplantation for Malignancies other than Hepatocellular Carcinoma

Attached is a request for a technology assessment that we need in order to develop a Medicare national coverage determination for liver transplantation for malignancies other than hepatocellular carcinoma. Please coordinate meetings, conferences, and deliverables through Carlos Cano at (410) 786-0245.

We are available to work with your staff throughout the course of the technology assessment. Please have them contact Jackie Sheridan at 410-786-4635 or Madeline Ulrich, MD, at 410-786-1591 with any questions.

Attachment

Technology Assessment Request Liver Transplantation for Malignancies other than Hepatocellular Carcinoma

Background:

Currently, Medicare national coverage policy for liver transplantation excludes coverage for all malignancies. In response to repeated concerns expressed by beneficiaries and physicians, we generated an internal coverage review on this provision. We found sufficient evidence related to hepatocellular carcinoma (HCC) to allow us to make a favorable decision. Attached is a copy of a draft of the decision memorandum we prepared on that issue.

The body of literature related to liver transplantation for patients with HCC includes several comparison studies of sufficient sample size to clearly establish patient outcomes comparable to liver transplantation for non-malignant conditions. It also demonstrates outcomes comparable to or better than those achieved by treatment of HCC by other means, such as resection and chemoembolization. However, our search of the literature did not produce a significant volume of literature, or literature of sufficiently sound methodology, to permit our staff to make a determination regarding liver transplantation for other malignant diagnoses, such as metastatic disease, bile duct carcinoma, and epithelioid hemangioendothelioma among others.

HCFA has determined that it does not possess sufficient internal expertise to fully and fairly review liver transplantation for these other malignancies, and that additional resources are required to facilitate this process. Accordingly, HCFA is

requesting that the Agency for Healthcare Research and Quality provide a review and assessment of directly and/or indirectly applicable literature for such disease entities.

Purpose and Scope:

The purpose of the technology assessment is to provide information to assist HCFA in determining whether there is sufficient evidence to conclude whether liver transplantation for malignant diseases other than HCC meets the criteria of being reasonable and necessary under the Medicare law. Further, the assessment should provide information to assist HCFA in determining whether there is sufficient evidence to conclude that it is appropriate to alter the existing national coverage policy with regard to treatment modality, and conclusions regarding the effectiveness of specific interventions.

The literature evaluation should include the materials resulting from the literature search as part of the assessment. If there are technology assessments that have already been performed, HCFA is requesting that AHRQ purchase and include in its assessment these assessments.

Search Strategy:

- Medline MeSH Headings – liver transplantation/ct (contraindications), liver transplantation/mo (mortality), liver neoplasms, and biliary tract, and carcinoma
- Cochrane Collaboration Systematic Reviews
- AHRQ data bases of international technology assessments
- In selecting literature for evaluation, consider applicability to the Medicare population (aged, disabled, or end-stage renal disease), and the clinical reputation, face validity, and the stature of the peer reviewed journal. Clinical trial and observational studies should be included.

Evaluation:

The primary issue to be investigated is whether or not the scientific and clinical literature resulting from the review support the use of liver transplantation as treatment of carcinoma other than hepatocellular carcinoma. Specific questions to be addressed include:

- Is there evidence that patient survival and/or disease-free survival of patients with carcinoma other than HCC is improved with transplantation as compared to other forms of treatment?
- Is there evidence that patients with carcinoma other than HCC survive as well with liver transplantation as do patients with other forms of end-stage liver disease?
- Are there characteristics or subpopulations of persons with carcinoma other than HCC whose outcomes with liver transplantation are roughly equivalent to other forms of end-stage liver disease? If so, what are they?
- Are there characteristics or subpopulations of persons with carcinoma other than HCC whose outcomes with liver transplantation are superior to those resulting from other treatment alternatives?

Based upon our preliminary review, we believe that the body of clinical trial evidence in this regard may be minimal. Therefore, the evaluation should include observational as well as clinical trial studies. We request that all of the studies produced from the review be critically examined with respect to inferential validity, possible sources of bias, and related factors. The classes of response variables of interest may include mortality (survival times), morbidity (complications), and quality of life (activities of daily living).

Search Time Constraints: 1990 and Later

Non-English Literature: No

Interim Meetings and Reports:

We are requesting that AHRQ meet with HCFA prior to the initiation of the assessment to discuss the purpose and study approach, scheduling issues, and follow-up interim meetings to discuss study results and report formats.

The following interim reports are requested:

Literature Review Results
Draft Report
Final Report

In accordance with our *Federal Register* Notice of the process for making Medicare national coverage decision (64 FR 22619), the final report will be posted on the Internet.

Time Frame: Six Months or Less